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Do Decision Makers Really Need Health Economic Data?

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In this issue of *Value in Health*, the ISPOR Task Force on Use of Pharmacoeconomic/Health Economic Information in Health Care Decision Making presents a report with information intended to ensure that pharmacoeconomic/health economic (PE/HE) research is attuned to the needs of decision makers and to provide tools they can use to interpret and apply these studies [1]. After reviewing the literature on decision makers' attitudes toward PE/HE evaluations, the task force has identified a number of key decision-maker concerns that may limit their use of results from PE/HE studies. In addition, following a review of published guidelines and discussions with managed care pharmacy opinion leaders, the task force suggests some additional reporting requirements to address these concerns.

Despite the authors' recognition that a number of issues remain to be addressed, these recommendations represent a step forward and add to the progress that has been made toward bridging the gap between PE/HE practitioners and decision makers. In recent years, an increasing number of managed-care organizations and pharmacy benefit managers have expressed interest in obtaining PE/HE data and many have sought training to aid their evaluation of these studies. Some organizations are using checklists to quickly assess the quality of PE/HE evaluations and developing relationships with consultants to assist in more extensive reviews. Through use of the AMCP Format, numerous organizations have sought PE/HE data to better inform their P & T decision-making processes [2].

Our organization began using the AMCP Format shortly after its inception in October 2000. We conducted an orientation session for our P & T Committee in early 2001 to discuss the Format and were encouraged to see how eager the members were to begin receiving this information in a more consistent manner. During the past 2.5 years, the Format has been a helpful tool for our pharmacists who prepare new drug monographs and has become ingrained in our P & T process. I was therefore surprised when our P & T Committee chairperson recently expressed disappointment owing to our lack of progress in this area. Despite the early promise of dossiers for new products, he was frustrated that very little PE/HE data from dossiers had been presented to the P & T Committee. Had we given up on the process? If not, where were the other dossiers?

My answer provided him with a new perspective on our processes and may shed some light on the disconnect between producers and consumers of PE/HE information. While there have been some exceptions, most manufacturers do provide an AMCP Format dossier. Unfortunately, however, many provide only a partial submission in which the sections on economic studies and modeling are blank. Of those who do provide economic models, many choose a framework that is not supported by the clinical data. In some cases, a product has some clinically meaningful advantages relative to existing products, but the model addresses only acquisition cost and rebates. These models offer little value because we already conduct this form of analysis as part of our routine internal process. Additionally, these models miss the opportunity to address the health improvements to be attained with use of the new product. In other situations, manufacturers have a more positive impression of their product than we do following our review of the available data. Here we find cost-effectiveness analyses driven by changes in clinical endpoints that may be statistically significant but not necessarily clinically meaningful. To ensure the efficient use of time, we have instructed our staff to provide only a cursory review of cost-effectiveness analyses that are based solely on clinical trials in which a meaningful clinical advantage has not been demonstrated. As a result, despite the receipt of numerous AMCP Format dossiers, we have highlighted modeled results from the dossiers in presentations to our P & T Committee on only a few occasions.

While some would argue that we have unrealistic expectations, we believe that we are taking a very practical approach. We are not seeking incredibly complex analyses, but rather straightforward evaluations of relevant questions. Yes, we want cost-effective drugs, but they must be affordable as well. The task force report mentions that some decision makers may have difficulty understanding the

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concepts behind willingness to pay. This may be true, but payers are well attuned to the willingness of employer groups to pay for rapidly increasing health-care premiums. They see the revealed preferences of clients forced to forego dental coverage for their employees to continue to offer a pharmacy benefit. Payers who see the rolls of the uninsured growing in the United States are beginning to take a closer look at reference pricing and other measures as alternatives to no coverage at all. In these circumstances, it should not be surprising that, for example, decision makers show only moderate interest in a comparison of the cost-effectiveness of various ARBs, while focusing much more attention on finding ways to maximize the appropriate use of ACEIs and thiazide diuretics.

As we seek to realize the benefits of PE/HE research in an environment of double-digit spending growth for pharmaceuticals [3], we need to continue to foster the conduct of quality research and to invest in the resources needed to interpret it correctly. Equally importantly, however, producers and consumers of PE/HE data alike need to be active in applying these principles to influence decisions within our respective environments. In many cases, this may require changes not only in the way decisions are made about specific therapies, but also in the way the business of health care is conducted.

In the payer environment, we need to encourage the appropriate use of safe and effective therapies and to support the careful consideration of various forms of evidence. Payers will need to ensure that their staff has sufficient training to evaluate PE/HE data or arrange access to consultants who can help. Increased dialog with those who produce PE/HE research will be needed to improve the relevance of modeled results. In view of the limited resources available, payers need to encourage the use of an affordable mix of products with proven value and recognize that, in many cases, important health improvements will require expansion of the drug budget. Many PBMs need to change their business model to begin to align their incentives with those of their clients and improve transparency around revenue sources, such as rebates and program fees, which might influence product selection.

To achieve success in this area, individuals who produce PE/HE data must also play an important role in applying these principles. To facilitate the cost-effective use of new and existing therapies, academics, consultants, and employees of pharmaceutical companies should lobby for changes in the way these companies study and promote their products. A few opportunities include:

- Clinical trials should address not only the needs of the FDA, but those of patients, providers, and payers as well. Intermediate endpoints, narrowly defined patient populations, and comparisons with placebo are important for registration, but provide little basis for comparative value assessments. Products are seldom cost-effective in all uses, yet payers seeking to reimburse a product when used in its most cost-effective manner often must instead make an all-or-nothing decision because data about its use in relevant subpopulations are lacking. Manufacturers should provide the data necessary to demonstrate the value of their products at launch rather than ask payers to assume the intrinsic value of new products while waiting for confirmation from a phase IV study. Findings from ALLHAT [4] and the Women's Health Initiative [5], along with the ongoing debate about the relative risks and benefits of COX-II inhibitors, serve as clear reminders for payers to seek confirmation of value before reimbursement. In response to insufficient data at launch, many health plans have imposed a standard delay period before reviewing new products.
- PE/HE analyses should be integrated early in the product development cycle to guide study design and influence pricing decisions. Many of the currently conducted PE/HE analyses appear to be an afterthought or addendum to clinical trials. Because PE/HE studies are dependent on the design and results of clinical trials, the relevance of PE/HE research is hindered from the outset. Although researchers may do excellent work despite these limitations, payers often view the endpoints chosen and the frequent assumptions required as an indication that these analyses are designed solely for post hoc justification of pricing decisions.
- Payers do not need bootstrap confidence intervals, acceptability curves, or bayesian analyses to be convinced that generic products represent clear cost-saving alternatives to their brand name counterparts, and yet multiple patent listings and lawsuits often needlessly delay their market entry. "Dramatic" improvements in the form of metabolites, enantiomers, or new delivery systems are suddenly achieved just before patent expirations of blockbuster products. Some pharmaceutical company Web sites tell consumers that they may experience different effects with a generic drug relative to the originator product despite the FDA's clear statements to the contrary.

- Direct-to-consumer advertising typically does an excellent job of educating consumers about the existence of a product and appealing to their sense of need, but little to encourage careful consideration of its value relative to existing comparators.

My background includes employment in the pharmaceutical industry and I recognize that many of my colleagues who perform PE/HE research to demonstrate the value of pharmaceutical products work diligently to prepare scientifically rigorous analyses that will meet payer needs and encourage the use of products with proven value. I applaud their effort. Careful consideration of the methodological standards set forth in existing guidelines, along with the additional reporting requirements included in this ISPOR task force report, should lead to improvements in the reliability and relevance of PE/HE studies. However, payers do not consider clinical and economic evidence in a vacuum, but rather, within the broader context of all promotion efforts of the industry. Thus, the credibility of even the best PE/HE research available may suffer if it is produced or sponsored by a manufacturer whose business practices demonstrate inconsistent support for, and often seem to be at odds with, the appropriate use of cost-effective and affordable therapies.

Much progress has been made to improve the conduct and reporting of PE/HE data. But while payers have expressed increasing interest in PE/HE data in recent years, many decision makers lack formal training in PE/HE and little is known about how, or if, they are actually using the information. Some have suggested that managed care "just doesn't get it" and that with sufficient training and cajoling, someday they will see the true merits of cost-effectiveness analysis and quality-of-life research. I submit that there is an alternative explanation—namely, that much of the current PE/HE information being presented to managed care is to some extent unnecessary, because the product has not been shown to offer a clinically meaningful advantage relative to existing alternatives; insufficient, because it does not give adequate consideration to affordability; or lacking the credibility necessary to adequately meet the information needs of payers. I believe that until progress has been made on these issues, further standardization of methodologies and reporting requirements and more training for decision makers will fail to achieve the desired objective.

References

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